THE HEMOPHILIA FOUNDATION
OF NORTHERN CALIFORNIA

A NATIONAL HEMOPHILIA FOUNDATION **MEMBER**

January 23, 2002

Jo Anne B. Barnhart
Commissioner
Social Security Administration
P.O. Box 17703
Baltimore, MD 21235-7703

***RE: RIN 0960-AD67***

Dear Commissioner Barnhart:

The Hemophilia Foundation of Northern California along with the National Hemophilia Foundation, hereinafter referred to as HFNC and NHF, is a not-for-profit organization dedicated to improving the quality of life for all individuals with hemophilia and other bleeding disorders. HFNC in collaboration with NHF wishes to provide written comment to your proposed revisions to the medical criteria for evaluating eligibility for federal disability assistance for patients with hemophilia and von Willebrand disease (vWD), which were published in the November 27, 2001 *Federal Register*.

While the advent of factor replacement products and comprehensive care models for the treatment of hemophilia and vWD patients have increased quality of life and reduced reliance on public disability assistance for many individuals, both adults and children, the continued importance of access to disability assistance cannot and should not be underestimated. We commend the Social Security Administration **(SSA)** for its past efforts to recognize the changes in the standard of care for the treatment of individuals with bleeding disorders and support the efforts of the **SSA** to reorder and regroup hematological disorders apart from malignant neoplastic diseases. While we commend the SSA for its commitment to updating its medical criteria in light of advances in the treatment of hemophilia, vWD and other diseases, we are concerned that these revisions are based on older listings that were not consistent and did not reflect the standard of care for persons with hemophilia. Thusly, many of the provisions in the proposed rule continue to reflect a poor understanding of the current standard of care for these disorders, and continue to exhibit inconsistencies. Furthermore, we are also deeply concerned

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about changes to the listings that could significantly reduce the ability of children with severe hemophilia to obtain disability benefits.

Proposed *7.00E—How* Do We Evaluate Episodic Hematological Disorders? Under this new section, an individual with hemophilia would be required to have bleeding episodes occurring at least 3 times in a consecutive 12-month period. These events can include "pain crises, hospitalization, treatment with parenteral antimicrobial medication, bleeding episodes and thromboses." Furthermore, the new section requires a respite of at least one month between health events to ensure that the agency is evaluating separate episodes. In the most severe form of the disorder, patients may suffer from frequent bleeding episodes and often these episodes can occur without a known injury. Therefore, requiring a month-long lapse between bleeding events to ensure that the agency is evaluating separate episodes may fail to recognize the course and severity of this disorder in a particular individual. A series of three separate, discrete bleeding episodes in a consecutive year may be less lifethreatening or disabling than a continuous, serious bleed. Also, the listing contends that a bleed must occur despite prophylactic treatment. While NHF does encourage the prophylactic treatment for patients with hemophilia (MASAC Recommendation #117, attached), *prophylaxis* is not a universal treatment and therefore should be removed from the SSA listing.

Proposed *7.00G(3)(a)—Disorders of* Hemostasis. This new section indicates that the agency will document "the frequency, severity, and treatment of bleeding episodes" as part of its effort to determine eligibility for disability assistance; however, the provision fails to define terms such as "frequency" or "severity." How does the agency reconcile the use of the term "frequent" with its requirement that a month transpire between bleeding episodes to determine if a bleeding episode is a new versus a recurrent bleed? How will the agency define "severe" and what documentation will be necessary? The Centers for Disease Control and Prevention's planning workbook on hemophilia indicates that bleeds in several key anatomical sites should always be viewed as potentially lifethreatening. These include bleeding into the head, neck or tongue, spinal cord or digestive tract. While bleeding into the head accounts is a leading cause of premature death in hemophilia patients, the symptoms of bleeding into the head, if present at all, can be diffuse, such as fatigue, headache and neck pain. Head bleeds may also occur spontaneously and may not be linked to either major or minor trauma to the head. We would urge the agency to use great caution in developing documentation criteria to accompany this section.

Proposed *7.00G(3)(c).* HFNC supports the agency's proposal to consider such complications as the development of inhibitors against clotting factor, intrusiveness of treatment, and joint deformity in its criteria for considering eligibility for disability. HFNC also encourages the agency to consider other complications such as bleeding-related nerve compression, which could lead to loss of cognitive ability as well as mobility and motility. The SSA should consider the confluence of multiple morbidities in individuals with hemophilia who are also HIV and/or HCV positive. The SSA should also be aware of other more rare complications such as the hemophilic pseudotumor, which if undiagnosed or untreated can lead to neuropathy, bone necrosis, and if located in

the large muscle masses of the pelvis may lead to erosion into the bowel. While the prevalence of this complication has decreased over the years due to the availability of adequate factor treatment, pseudotumors continue to be considered a significant complication of hemophilia, especially in HIV-positive individuals with hemophilia. NHF also encourages the agency to consider the complications of treatment, such as the development of .hepatitisafter the use of plasma-derived products. While advances in technology have significantly reduced the risk for contracting viral infection from plasma-derived products, the risk for contracting blood-borne viruses still exists. Indeed, the recent shortage of recombinant product has led to an increased reliance on plasma-derived products, further increasing the risk of contracting hepatitis. While vaccines currently exist for hepatitis A and B, no vaccine exists for hepatitis C, and hepatitis C infection represents a growing problem in the hemophilia community [This implies that hepatitis C infections continue to occur; in reality, there have been no new infections in over 10 years from plasma-derived products]. Furthermore, hemophilia **patients are** at risk for contracting other infectious diseases through the use of plasma-based products. While donor deferral programs are currently being implemented and the exact risk for contracting Creutzfeldt-Jakob Disease from blood products is unknown, hemophilia patients may be at risk for developing this lethal disorder through the use of plasma-derived replacement factor. Persons with hemophilia are also at risk for developing parvoviruses, which cannot be "killed" using current methods for killing viruses. While very common in the general population, parvoviruses may pose a more significant health risk for those hemophilia patients with immune systems compromised by HIV or HCV infection. Finally, the SSA needs to consider disability caused by the use of essential medications such as HAART, interferon, ribavirin and certain narcotics.

***Proposed** 7.03B—Category of Impuirments, Disorders of Hemostasis.* The HFNC's concerns with the language of 7.03B were previously stated in our comments to section 7.00E.

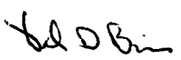
***Proposed** 7.03C—Category of Impairments, Disorders of Hemostasis.* HFNC **is** dismayed with the provisions in section 7.03C that outline the eligibility criteria for vWD, and would ask that the agency hold patients with this disease to the same criteria, i.e. number of bleeding episodes per 12-month period, as in hemophilia. vWD is reported to be the most common bleeding disorder in humans. Although the disease affects both sexes, women with vWD are at higher risk for complications such as anemia, because of menses. Section 7.03C states that a hospitalization of 24 hours or more, occurring at least 3 times in a consecutive 12-month period is necessary for consideration **of** disability for a patient with vWD. HFNC opposes in the strongest terms this criteria as it sets a higher standard for individuals with vWD for disability assistance than it does for any other hematological disorder. vWD is classified into three major types: I, 11, and 111. Type II vWD is subdivided into four different subtypes. Correct typing of vWD is necessary to ensure that the proper treatment regimen is followed. People with type I vWD are treated with the synthetic agent desmopressin, either as an injectible or in nasal spray form. In the case of surgery, trauma or other serious bleeding episodes, a factor VIII concentrate with a high concentration of von Willebrand factor is often required. Persons with type IIA, IIM, and IIN are also treated with desmopressin. Individuals with the more severe

forms of the disorder, IIB and III or those non-responsive to desmopressin are treated with factor VIII concentrate that contains a higher molecular weight multimers of von Willebrand factor. Because of the increased risk of HIV and hepatitis transmission, cyroprecipitate is not recommend except in the direst of emergencies. The standard of care for individuals with vWD, as with hemophilia, does not require routine hospitalization. Furthermore, the SSA recommendation goes against shifts in our health care delivery system away from in-patient care to treatment in an outpatient facility or at home.

***Proposed Listing 107.03—Disorders of Hemostasis (in children).*** SSA proposes to utilize the similar criteria for hemophilia and vWD in children that it will use in adults (see. 7.03B and C). The SSA contends that these changes will bring clarity to the issue of children's disability. Certainly, the previous listing was confusing both to parents and to SSA staff, resulting in variability ~~from state to state in disability determinations~~. While we support the intention of bringing clarity to this issue, we strongly object to proposed changes that would specify the level of joint deformity required in children with hemophilia, which is not a requirement for adults, using the criteria in listing 101.02, the listing for juvenile rheumatoid arthritis (JRA). The etiology, pathophysiology and treatment for JRA and hemophilia-relatedjoint disease are vastly different. JRA is an autoimmune disease characterized by swelling and stiffness and limitation of movements in the joints. In addition, in JRA high fever and skin rash are often present. JRA is diagnosed through sero-testing for rheumatoid factor and antinuclear antibodies. Treatment regimen for JRA includes heavy use of non-steroidal anti-inflammatory drugs (NSAIDS), corticosteroids and other disease-modifling anti-rheumatic drugs. Hemophilia is predominantly an inherited disorder, although acquired hemophilia does exist. The breakdown in synovium in the joints of persons with hemophilia is due to repeated bleeding into that joint, whether from minor or major trauma or from spontaneous bleeding. While physical therapy is recommended as a treatment for both disorders, the most common treatments for minor hemarthroses include infusion of factor, immobilization and elevation, compression and ice therapy. The use of many analgesics commonly used to treat JRA, including aspirin and NSAIDS, are contraindicated because of the risk of potential bleeding due to inhibition of platelet function. In more serious cases, surgical intervention may be warranted.

I hope that these comments will be useful to the SSA in revising its criteria for disability. NHF would welcome the opportunity work with the agency to provide you with expert knowledge on hemophilia and other bleeding disorders and we look forward to further discussions on the proposed listing.

Sincerely,



Val D. Bias
President HFNC